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Message from the Executive Director

Do things ever get less busy? For any of us? Like many of you, the phrase “when we have spare time” has become a wry motto for the staff, board, and volunteers that drive the IPPF forward.

And so, what do we do when these things all stack up? We have productivity tools, but just like you, we prioritize, combine, and try to work both harder and smarter. We are always trying to expand our reach in support of the goal that “Biopsies Save Lives.” To support this, we try to talk to as many practicing and student medical professionals (doctors, dentists, nurses, hygienists) as we can so that they consider the possibility of pemphigus or pemphigoid as a diagnosis when examining patients. We have had considerable recent success in doing this. Through a partnership with the American Dental Association and Paradigm Medical Communications, Donna Milton, MD, PhD; Joel Laudenbach, DMD; and our own Becky Strong, BS, RN (Outreach Director), recently presented a live continuing education course called, “Pemphigus: Know it when you see it.” While this is part of what we regularly do, this time there were almost 3,000 registrants!

In addition to this, staff members Becky Strong and Amethyst Yale (Outreach Assistant), continue to support a higher number of Patient Education Series webinars in 2021 after we increased the frequency of these webinars in response to the COVID-19 pandemic. They are ably supported by Patrick Dunn (Communications and Marketing Director) and Anna Lane (Communications and Marketing Manager) as we also promote the IPPF’s initiatives and successes, echo those of others, and produce compelling content—including this very publication! Meanwhile, Nelly Filippov (Administrative Manager) keeps us moving forward and regularly steps in to remedy my own administrative and record-keeping shortcomings.

And our volunteers—wow! If I were to name a few, I would leave too many out, but suffice it to say that our efforts in peer health coaching, research, advocacy, event planning, and strategic direction benefit enormously from the active participation of volunteers. Related to this, please join me in welcoming three new members to the IPPF Board of Directors: Carolyn Fota, Laurence Gallu, and Michael Rigas. The entire community is looking forward to their contributions in these important roles.

In the past quarter, we have heightened our advocacy work to support various federal level legislative efforts that impact quality of life and affordability concerns of patients. As one of the stronger rare disease organizations, we also lend our support to other organizations with similar goals around health access. In the US, Rare Disease Week on Capitol Hill is two months away, and we will be playing our usual prominent role in that. As always, we are looking forward to continuing our efforts to support the pemphigus and pemphigoid community in the months ahead.
The US Food and Drug Administration (FDA) held a Listening Session with patients representing the International Pemphigus & Pemphigoid Foundation (IPPF) on February 8, 2021. Patient Listening Sessions are intended to be an opportunity for the FDA’s medical product centers to engage with patients and their advocates. The IPPF session was patient-led, meaning that the IPPF requested and received the permission to share its members’ perspectives with the FDA.

Session Objective
The objective for this listening session was to have a dialogue with the FDA to share the emotional experience of the patient journey as well as the burden these diseases have on all aspects of a person’s life. This includes the time it takes to get a diagnosis, the burdens of treatment options, and the undertreated areas of the diseases that affect the physical, emotional/psychological, and financial health of five pemphigus and pemphigoid patients.

Summary of Topics Discussed
Pemphigus and pemphigoid are rare, ultra-orphan, autoimmune, blistering diseases that result in potentially life-threatening destruction of the skin and mucosa. The patient’s immune system makes antibodies that attack healthy cells in the skin or mucous membranes. As a result, skin cells separate from each other, fluid collects between skin layers, and blisters form. These blisters may cover a large area of skin. This results in fragile, extremely tender lesions that do not go away without proper treatment. It takes the average pemphigus or pemphigoid patient five healthcare providers and ten months to obtain a correct diagnosis. Currently, no cure exists for pemphigus or pemphigoid, only treatments and remission.

According to recent literature in the *British Journal of Dermatology*, pemphigus is rarer than pemphigoid. The approximate incidence of pemphigus is 0.58 - 0.80:100,000 people, and the approximate incidence of pemphigoid is 7.3 - 7.93:100,000 people.

These diseases are known to affect people across gender, racial, and cultural lines. However, there are
certain groups of people who have a higher incidence of the diseases, such as Eastern Europeans of Jewish descent and people of Mediterranean, Northern India, and Persian descent.

Diagnostic Delays
For a definite diagnosis, doctors consider the clinical presentation and visual exam of oral and skin lesions. Many times, a lesional biopsy is taken and examined under a microscope for traditional histology and exam (H&E). Additionally, the layer of skin in which cell-to-cell separation occurs can be determined. Frequently, direct immunofluorescence is used to look at a treated skin sample to detect desmoglein antibodies in the skin. Indirect immunofluorescence or antibody titer test is also used to measure the autoantibodies in the blood serum.

All patients described the journey from first seeking care to a final diagnosis of pemphigus or pemphigoid. Without exception, it took months to years to achieve diagnosis, with multiple practitioners being consulted and many instances of incorrect diagnosis. The IPPF commissioned a study in 2011 and found that the average patient had to see five different healthcare providers over the course of ten months to receive a correct diagnosis. Ten percent of respondents in that study reported having to see over ten healthcare providers to receive a correct diagnosis.

Each of the five patients who participated in the Patient Listening Session described how their disease started with benign symptoms that were explained away as oral canker sores, a sore throat, a bug bite, tonsillitis, or other common problems. Patients described a vast decrease in their quality of life and a loss of independence as the disease progressed and their health deteriorated. As members of the military, a single mother, a pilot, and a young healthcare administrator, these patients were strong and independent, but they eventually had to move in with family members, rely on others for personal around-the-clock care, and even declare bankruptcy from losing the ability to work.

Working was difficult, and many with pemphigus and pemphigoid experience bias based on having skin lesions. The IPPF Founder described how she lost her job due to her skin lesions because she no longer looked clean and healthy. Another described laying in a hospital ICU wrapped up “like a mummy.” The financial burden of having pemphigus or pemphigoid is greatly related to a patient’s inability to work or to do their job in the same way. Patients are further burdened by using off-label treatments that are not part of an insurance formulary for pemphigus and pemphigoid.

Treatment Options
Pemphigus and pemphigoid are chronic illnesses that, with rare exception, do not improve without active treatment. Treatment approaches include a control phase, a consolidation phase, and a maintenance phase, with the possibility of complete remission or disease relapse (flare).

Initial therapy is determined by the extent and rate of the progression of lesions. This includes the control and consolidation phases of treatment. The priority is to control lesions. In a slow, progressive form of the disease, initial treatment usually includes oral and topical corticosteroids, as well as intralesional injections of corticosteroids.

In the consolidation phase, drugs and doses are maintained until complete clearance of lesions. Once most lesions have healed, the dose and type of medication are gradually reduced to limit the risk of side effects. This is the maintenance phase. The rate of dose reduction is determined by clinical response and overall disease activity. It is important to monitor this balance and limit use of unnecessary medication as many fatalities are related to complications with therapy.

Relapse may occur at any time, resulting in renewed disease control efforts.

Currently, Rituximab is the only FDA approved therapy for moderate to severe pemphigus vulgaris. Other off-label therapies include immunosuppressants, intravenous immunoglobulin, and anti-inflammatory agents.

Investigational therapies currently being researched include monoclonal antibodies, Anti-C5aR Antibody, CAAR-T cell therapy, BTK inhibitors, FcRn receptors, and other recombinant small proteins that reduce the immune/inflammatory response.

All five patients shared the treatment plans they were given upon their diagnosis of pemphigus or guidelines due to the dose of prednisone he had been prescribed. Another described laying in a hospital ICU wrapped up “like a mummy.” The financial burden of having pemphigus or pemphigoid is greatly related to a patient’s inability to work or to do their job in the same way. Patients are further burdened by using off-label treatments that are not part of an insurance formulary for pemphigus and pemphigoid.
pemphigoid. Prednisone was consistently used as an early treatment, and it is also still being prescribed as a part of step therapy. The doses stated were high—up to 0.5 - 1 mg per kilogram of body mass. This created additional physical and mental health issues, such as weight gain, anxiety, depression, social isolation, hypertension, and mood swings.

Further treatments often involve off-label usage, leading to both delays in prescription and insurance coverage issues.

Two of the five patients who participated in the Listening Session were not in remission, but they both felt that their prior experiences, coupled with an increase in available information and support, made it easier to cope with flares.

**Medical Burdens**
All patients commented on the pain involved with their disorders. The pain associated with blistering was accompanied with pain from the dressing of blistering locations and, in some cases, by treatment associated with an incorrect diagnosis.

**Investigational Research**
The IPPF reviewed some of the major research and trials being conducted.

**Mental & Social Burdens**
Patients present had diagnosis dates as recent as three years and as distant as over thirty years. Those with a less recent diagnosis were often exposed to statements such as, “this disease is fatal,” in pre-internet sources.

In many instances, the disorder was noted to be disfiguring. This caused alienation and a feeling of shame in some cases. Once blistering was controlled, there was still the potential for long-term scarring.

All patients reported the opinion that these conditions could not be faced alone. All had a support system beyond the medical practitioners involved in their care. For many, work became difficult or impossible, with the associated shortfalls in income and insurance coverage. Additionally, some treatments required infusions that took 7 - 8 hours to administer and had side-effects that lessened patients’ ability to work.

**FDA Divisions Present**
- Center for Drugs
- Center for Biologics
- Center for Medical Devices
- Reagan-Udall Center
- Office of the Director

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<th>Patient</th>
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<td>5</td>
<td>Bullous Pemphigoid</td>
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**Disclaimer**
Discussions in FDA Rare Disease Listening Sessions are informal. All opinions, recommendations, and proposals are unofficial and nonbinding on the FDA and all other participants. This report reflects the IPPF's account of the perspectives of patients and caregivers who participated in the Rare Disease Listening Session with the FDA. To the extent possible, the terms used in this summary to describe specific manifestations of pemphigus and pemphigoid, health effects and impacts, and treatment experiences reflect those of the participants. This report is not meant to be representative of the views and experiences of the entire pemphigus and pemphigoid patient population, or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.
IPPF

NATURAL HISTORY STUDY

A patient registry that uses an online data system to collect, store, and retrieve patient data for analysis in research studies.

Register at:
www.pemphigus.org
The IPPF is assisting in the planning of a Scientific Meeting and Satellite Symposium to the 50th Annual Meeting of the European Society for Dermatological Research (ESDR) on September 19, 2021. Along with the IPPF, “Pemphigus and Pemphigoid: Lost in Translation?” is being organized by Dario Didona, Rudiger Eming, Jens Waschke, Barbara Horvath, Luca Borradori, and Michael Hertl. IPPF Executive Director Kevin Mead interviewed Michael Hertl about the significance of the meeting and the PEGASUS research project being conducted in Germany.

Kevin Mead (KM): Why do scientists, researchers, and clinicians need to meet?
Michael Hertl (MH): Bullous diseases, and the research into them, are global. There are multiple sites involved in research here in Germany, and the same is the case in the US. Elsewhere, from Japan and China to Iran and from Finland to Spain, great work is being done.

We need to get together and communicate and discuss the research, the findings, and the possible applications to therapy development. These frank and open discussions are also a part of the requirements of the PEGASUS project—part of the funding for the project is to provide a subsidy for this symposium. The PEGASUS project is “a consortium of basic researchers and clinician-scientists with a long-standing interest in pemphigus and immunological research who will aim at dissecting key events in the immunological induction and maintenance phase of pemphigus vulgaris.”

KM: Can you give more details about the PEGASUS project?
MH: PEGASUS is a project with funding from the German equivalent of the US National Institutes of Health. The overall goal is to better understand the immunological regulation of the production of IgG autoantibody production, and to look for indications as to how this can then be translated into clinical practice with patients. Moreover, the physiology and pathology of epidermal adhesion is studied in detail. It is designed to look at pemphigus in three stages:
Stage 1: Cellular (immunology and pathogenesis)
- T and B cell interactions
- Immune factors
- Innate immune factors
- Physiological and disturbed epidermal cell adhesion

Stage 2: The mouse model
This then builds into an animal model.

Stage 3: Clinical practice
There are six centers across Germany that participate in this project, including clinics and universities here in Marburg, Berlin, Aachen, Giessen, and Munich.

KM: From the patient perspective, why are these meetings like the one scheduled for September important?
MH: The way the disease appears in humans—flares, triggers, treatment failures, as well as the hopes and fears of patients—cannot be created in a laboratory or using the mouse model. All of these things need patient input. But clinicians are not trained to operate within a laboratory, and so questions and issues presented by clinicians and their patients need to be brought into the laboratory of exploration and a search for answers.

Any research needs to have basic researchers who are excited about the disease and about the possible impact that they can have on patients. By meeting in September, we can provide both ideas and encouragement to emerging researchers.

KM: How is research on pemphigus and pemphigoid (P/P) both related to and different from research on other autoimmune diseases?
MH: Within the PEGASUS project, we are looking at pemphigus not only to research the disease itself and to find therapies, but also because the well understood pathogenesis of pemphigus means that it can act as a “model” for research into other organ-specific autoimmune diseases.

Research into CAAR T-cells is a growing field, and as with any research the goal is to develop therapies that can be carried into the clinical setting to effect real change with patients. In Marburg, a phase I trial with nanoparticle-based delivery of autoantigenic peptides aimed at targeting autoaggressive immune (T) cells in pemphigus is currently in place.

The “orphan” status of P/P means that regulatory approval times can often be accelerated. This means a shortened time-frame from research to clinical practice, which is both beneficial for patients and makes such diseases attractive for researchers who want to see patient benefits from their work.

KM: It is unfortunate that we will not be able to get together in-person in Marburg this September. [Staff members from the IPPF will not be traveling to Marburg this September due to the COVID-19 pandemic.] What would we have seen if we were there?
MH: Marburg is the home of the oldest protestant university and is an ancient medieval city. But it has modern scientific relevance too—the diphtheria vaccine was produced using horses in the stables at the university. The very first Nobel prize for medicine was granted for this in 1900 to Emil von Behring. More recently, Marburg is the manufacturing site for the BioNTech vaccine against COVID-19 in Europe. If we were able to meet in person, our opening ceremony would have been in the museum and we would have had a dinner in the castle.

This meeting was always designed to be a lead-in event to be held immediately prior to the ESDR meeting in Amsterdam. When that meeting went virtual, that had to be our decision as well. I hope that as things normalize, we will be able to welcome you all to Marburg to enjoy the historical town, the traditional university, and the lively presence with many students from all over the world.

Dr. Michael Hertl is a professor in the Department of Dermatology and Allergology at Philipps-Universität in Marburg, Germany. He is a board-certified dermatologist and immunologist and has trained several years in basic research laboratories at Case Western Reserve University, Cleveland, OH, and the National Institutes of Health, Bethesda, MD.

Kevin Mead joined the IPPF as the Executive Director in October 2020. He has over 25 years of experience in association management, fundraising, and project management.
The National Economic Burden of Rare Disease Study Report

A STATEMENT FROM THE EVERYLIFE FOUNDATION FOR RARE DISEASES

The National Economic Burden of Rare Disease Study report was released on February 25, 2021. This study, conducted by the Lewin Group on behalf of the EveryLife Foundation for Rare Diseases, is the first of its kind, providing the most comprehensive assessment of the total economic burden of 379 rare diseases in a single year. This study identified direct medical costs, via an analysis of claims data, and indirect costs associated with productivity loss and non-medical and other uncovered healthcare costs, via a survey of 1,399 members of the rare disease community.

A February 25 press release from the EveryLife Foundation announced the study and acknowledged the tremendous collaborative effort, most notably from the rare community ourselves, required to bring the study to life. Topline findings were presented at the Rare Disease Congressional Caucus Briefing on the same day in a presentation titled, “Economic Burden of Rare Diseases in America: A Public Health Crisis.”

The study found that the total economic burden of 379 rare diseases was nearly $1 trillion in the United States in 2019, exceeding estimates for many of the country’s most prevalent chronic diseases. One of the study’s most compelling findings is that excess direct medical costs associated with a rare disease diagnosis are not the largest cost driving category. Indirect costs associated with productivity loss are the largest cost driver, coming in at $437 billion dollars and representing 45% of total costs. The third cost category assessed, non-medical and uncovered healthcare costs, came in at $111 billion. These two cost categories are particularly significant to the rare disease community because these are the cost categories that have never before been quantified and yet represent the costs that fall directly on patients and families.

The report estimates that 15.5 million individuals in the US have any of the 379 rare diseases included in the study. These numbers underscore both the massive public health crisis of rare diseases, and the need for additional research and funding. By funding research, improving awareness and diagnosis, and supporting legislation, we can begin to relieve the massive economic, medical, and personal burden of rare diseases.

The EveryLife Foundation for Rare Diseases is encouraging each of us to contact our elected representatives, share the study findings, and urge Congress to support important rare disease appropriations priorities that would advance critical rare disease research and therapy development at the National Institutes of Health, the Centers for Disease Control and Prevention, and the Food and Drug Administration. Thank you to IPPF community members for participating in this survey. The full study report is available at: www.burdenstudy.org.
The International Pemphigus & Pemphigoid Foundation has published a white paper, “Elevating Awareness of the Pressing Needs of the Pemphigus Community,” in response to the results of a Patient Forum held on October 2, 2020, as part of the Virtual Patient Education Conference: “Creating a Brighter Future Together.” The Patient Forum was sponsored by Principia Biopharma, a Sanofi Company, and brought together pemphigus patients, caregivers, patient influencers, healthcare professionals, thought leaders, and advocates to discuss the most important disease-related topics affecting the pemphigus community. The white paper discusses the consensus reached by the speakers and panelists of the Patient Forum related to the most urgent needs facing the pemphigus community, and it includes decisive calls to action.

During the Patient Forum, panelists discussed their experiences with delayed diagnoses and the physical, mental, and financial impact of pemphigus. The consequences of a delayed diagnosis to a patient’s health and well-being can be significant, therefore the speed and accuracy of diagnosis must be improved. Speakers discussed the high dosage use of corticosteroids and limited treatment options for pemphigus, which makes it clear that new treatment options are needed. In response to the need for new treatments, scientists in academia and the biopharmaceutical industry are currently working to refine and expand treatment options that are more tolerable and targeted.

Based on the urgent needs discussed during the Patient Forum, the panelists and speakers agreed on the following calls to action:

1. Elevate awareness of pemphigus and the patient journey and perspective among key audiences, including clinicians, thought leaders, government agencies, and lawmakers, among others.

2. Increase awareness of ongoing clinical trials of promising investigational candidates to improve participation in trials, with the goal of developing more effective and well tolerated treatments for pemphigus.

3. Improve affordability of and access to therapies.

Access the white paper for an in-depth analysis of the challenges facing pemphigus patients during both diagnosis and treatment: https://go.pemphigus.org/elevating-awareness-pemphigus-white-paper
Becky Strong

Over the past year, all of our lives seem to have been turned upside down. Many of us had to find new ways to do our job, including staff at the IPPF. We are first and foremost a patient support organization, but spreading awareness is also very important to the Foundation and our community. Our Biopsies Save Lives campaign is aimed at accelerating the time it takes patients with pemphigus and pemphigoid (P/P) to receive a correct diagnosis. We use a variety of strategies to increase awareness, including patient educator presentations at universities throughout the US, exhibits at targeted dental conferences across the country, continuing education course offerings, and extensive advertising.

In 2020, we were lucky to exhibit and present a continuing education course at a major dental meeting in January. We also sent patient educators to three universities before COVID-19 shut down the world in March. Prior to the start of the pandemic, we were used to traveling to universities and dental conferences in person. With that no longer being a viable option, we had to figure out new ways to raise awareness of P/P and shine a light on our diseases.

The IPPF quickly adapted to the global pandemic and maintained our presence at dental schools by offering video-conference presentations at universities across the country. Despite the pandemic, IPPF patient educators shared their stories to 11 universities in 2020, reaching 1,260 dental students. In this way, we were able to provide patient interactions to students who were not able to see patients in person.

Additionally, the feedback from students after these virtual presentations couldn’t have been better:

*How long it took to get a diagnosis opened my eyes to the important role we play in the initial diagnosis process.*

*The speaker was amazing and was very open with sharing their personal experiences. She really drew from her own story to show us how an early diagnosis would have affected her life. She also gave realistic things to implement into our future practices (consider a biopsy, listen to the patient, reach out to organizations).*

*Medicine is a team effort. Treating patients is not about me; it’s not about how smart or talented I am. It is my job to find people to help my patient if I am unable.*

The IPPF also had to transition our continuing education activities in 2020. We were lucky to partner with Paradigm Medical Communications on continuing education courses and had planned to present at three major dental conferences. However, we were only able to present a live course in January, 2020, at Yankee Dental Congress in Boston. Through our collaboration with Paradigm and two of our medical advisors, we were instead able to virtually present at the Greater New York Dental Meeting in November 2020. Our wonderful medical advisors Dr. Donna Culton and Dr. Joel Laudenbach provided content about P/P symptoms, diagnosis, common treatments, and how dentists and dermatologists work together to treat P/P.
patients. The journey of IPPF Patient Educator Ellen Levine was used as a case study to demonstrate the impact of intervention and how her diagnosis could have been accelerated. Over 400 dental professionals took the course at the Greater New York Dental Meeting.

We also worked with Paradigm to create three free online CE courses: “More Than Skin Deep: Improved Strategies for Pemphigus Vulgaris Management;” “What To Do With What’s New In Pemphigus Vulgaris;” and “Know-It-When-You-See-It Diagnosis and Initial Treatment of Oral Pemphigus Vulgaris.” These courses are available on our website.

What does 2021 look like for the Biopsies Save Lives Campaign and the IPPF Awareness Program? We had hoped to return to in-person events; however, we are currently not able to do so.

Student Education

Instead, our patient educators continue to be busy presenting virtually to both dental and medical students across the country. So far this year, we have already presented to dental students at Harvard University, Temple University, Tufts University, and Rutgers University, as well as dermatology and interdisciplinary students at Emory University, SUNY Downstate, and Florida International University. We also have more presentations lined up for this summer and fall.

Professional/Continuing Education

This past April, I was invited to share my patient perspective and diagnosis journey as part of a continuing education course at the Dermatology Nurses Association Annual Meeting. I also shared tips and tricks that nurses can use with P/P patients to make dermatology appointments more comfortable.

Finally, we recently presented our most high-profile presentation to date: a continuing education course offered by the American Dental Academy (ADA). In addition to the live event, the 1.5-hour course will be available for the next year as part of the ADA’s enduring content.

We are looking forward to safely returning to in-person events when it is safe to do so. Whenever that may be, we plan to incorporate both in-person and virtual events to increase the reach of the Biopsies Save Lives and Awareness program in the post-pandemic world.

Becky Strong is the IPPF Outreach Director. She was diagnosed with PV in 2010 and is currently in remission. She lives in Michigan with her family.
Becoming an Advocate
TWO PATIENTS' PERSPECTIVES ON FINDING THEIR VOICES WITH RARE ACROSS AMERICA

Sharon Radtke

Almost 14 years ago, I moved to Vermont in order to live closer to my daughter and her family. A year later, I was diagnosed and successfully treated for melanoma. Soon after, I felt a burning sensation in my mouth, and that sensation lasted for eight years. In 2015, my first blisters appeared, and within three months I was diagnosed with pemphigus vulgaris (PV). Luckily, there was a dermatologist here in Vermont who had studied PV at Johns Hopkins University and knew what it was.

After my PV diagnosis, I began a regimen of prednisone, CellCept®, and rituximab. I experienced terrible side effects from the prednisone and had to use a cane to walk for six months. It took five years of taking CellCept® and eight rituximab infusions to reach remission. As a result of my PV and the challenging treatments, I could no longer continue my work as an event specialist because the work was too rigorous and stressful. Instead, I began taking pictures of birds as a distraction from the PV treatments, ended up discovering a new avocation, and created a wildlife photography business. Although I have been in remission for seven months, taking pictures is still what gets me through each and every day.

When the IPPF asked me to serve as an advocate during Rare Across America, I was not sure it would be a good fit for me. However, I remembered that throughout my lifetime I have been an outspoken medical advocate for my family, friends, and myself. I decided that particular skill might be useful in becoming a PV advocate.

Living in Vermont with PV has been challenging because there are 600,000 residents and only a handful of patients with PV. Our congressional representatives include US Senators Patrick Leahy (D-VT) and Bernie Sanders (D-VT), as well as US Representative Peter Welch (D-VT). I had previously met all three representatives through my events business, so I did not feel too intimidated speaking with their staff when asked to be an ambassador. In fact, it was quite easy. I told my story about PV and discussed the following:

- We need more education for dentists about PV so they can diagnose it sooner.
- Something must be done about the outrageous cost of medications, especially rituximab infusions, which cost $25,000 per infusion in Vermont. If I didn’t have medical coverage, I would have gone bankrupt eight times over.

During the Zoom advocacy sessions for Rare Across America, I met some very interesting Vermonters with rare diseases, and they taught me how to advocate. They discussed the latest rare disease bills being considered in the Senate and the House of Representatives. I also realized how much more I need to learn to be a successful rare disease advocate. The only thing that could have made my experience during Rare Across America better is if I could have met with my representatives in person, in Washington, DC.

All of us with pemphigus and pemphigoid have had to learn to be medical advocates for ourselves. Advocating during Rare Across America takes it one step further because we can influence legislation that could change our lives and those of patients who are diagnosed with P/P in the future. Becoming an advocate has helped me find my PV voice.

Sharon Radtke is a retired events and marketing specialist who was diagnosed with pemphigus vulgaris in 2015. She lives in Vermont with her husband and family and spends her time as a wildlife photographer.
Thank you to all of the IPPF rare disease advocates who participated in Rare Across America this past March. We had 10 advocates participate in 40 meetings! According to the EveryLife Foundation, there were more than 670 rare disease advocates that participated in 373 meetings with their federal legislators or staff members during the month of March. The IPPF is also looking forward to Virtual Rare Disease Week on Capitol Hill this summer from July 14-22. Learn more here: https://everylifefoundation.org/rare-advocates/rare-disease-week/.

Mindy Zimmerman

Until recently, I had not been a very active participant in politics or policy legislation. When I was diagnosed with pemphigus vulgaris (PV) in 2018, it was gratifying to learn about the International Pemphigus & Pemphigoid Foundation (IPPF) and the great educational and advocacy work they do. After my diagnosis, I became interested in educating others about my disease. Due to the high doses of prednisone and CellCept® during my first year, when I heard about Rare Across America in early 2019, I felt unable to attend due to my compromised immune system. However, by October 2019, I was able to attend my first IPPF Patient Education Conference. At that conference in Philadelphia, PA, I became more aware of how important my voice is, and I decided to become more active with the Foundation.

This past March, I participated in Rare Across America. The Every Life Foundation for Rare Diseases and the Rare Disease Legislative Advocates (RDLA) helped prepare me for an exciting first-time experience advocating for rare diseases. The RDLA provided me with many tools and helped me to plan for my meeting with my congressional representative. During the weeks before my virtual meeting, I had the opportunity to attend many educational webinars, and my team leader also guided me in preparing for my story and my “asks” for the meeting.

Marc Yale, IPPF Advocacy & Research Coordinator, also reached out to me to share information on specific legislation to present at my meeting, and he offered his guidance and support. By the time the meeting took place, I was ready to share my story about being a patient with a rare disease and ask my representative to cosponsor the Helping Experts Accelerate Rare Treatments (HEART) Act (H.R. 1184).

Meeting with my representative’s assistant and staff was a first for me. Throughout this process, I learned that building relationships with individual staff members is key. Such connections will continue to be important in my journey as a rare disease advocate at the national level. Now I know how I can help to make a change in the lives of rare disease patients and their families.

I also had the opportunity to participate in another advocacy day on the Hill on March 17, 2021, which was organized by the American Association for Dental Research (AADR) and friends of the National Institute of Dental and Craniofacial Research (NIDCR). It was a privilege to meet with the same staff members of my representative in Congress. I found out that my congressman signed on to co-sponsor the bill that I had asked him to support during Rare Across America. Wow, talk about instant gratification! I really felt like I had made an impact by sharing my story.

When I read that Rare Disease Week on Capitol Hill will be virtual this year from July 14-22, I signed up! I’m looking forward to continuing to advocate and make my voice heard this summer. I have learned that telling my story is important, and I can have a real impact by sharing it. I know that by establishing and maintaining relationships with my legislators, I will continue to make a difference.

Mindy Zimmerman is a PV patient who lives in South Florida. She is a saxophone and clarinet teacher and plays in a community band.
The last year has felt like a blur without the anchors of daily life, like getting in the car, going to work, leaving for lunch, or coming home. The moments in the car waiting for kids at school or running to the store for an item or two we needed were lost. With none of my anchors, it felt like I had lost my freedom. It was all seemingly gone.

Having a rare disease can strip you of a lot—time, power, and security. It can fill you with fear, anguish, and worry. Early on, I saw the pandemic coming. I was attentive to early news reporting, and I got my ducks in a row. Being diagnosed with pemphigus has taught me that I must advocate and watch out for myself.

In January 2020, when COVID-19 was discovered in Washington state, I had, somewhat robotically, added to my pantry. I didn’t know toilet paper would be so hard to find, but I had enough anyway. It was easy for me to rationalize stocking up on beans, canned tomatoes, and dried veggies. I could always donate them if I didn’t use them. Also, why not get a couple cases of broth cubes? That’s not obsessive, right?

By February, I tweeted with a woman stuck on The Diamond Princess cruise ship that was quarantining in Japan. She was named in a news report that talked about the ship’s predicament, and I reached out. I thought about how hard it must be for everyone on the ship. I couldn’t imagine. She told me how the virus spread and infected people. And the pandemic began ….

I carried a little extra Purel, kept more distance from other people, and used cleaning wipes on shared surfaces. A coworker laughed at me when I carefully used my sleeve to open a door for a meeting. It was infuriating, and I felt humiliated. Maybe they were in denial? Maybe I was losing it?

I wrote to our rabbi in early March and asked him how we could help our congregants and amass a volunteer army:

My feeling is and has always been . . . if I am well enough, I should spread that love and strength. I have a rare autoimmune condition, and I work hard to help educate the
medical community about it because I know other people aren’t able to do that. The same goes here for me, I can always make some for you. It’s something. And it says we care and we are here. Together, I think we can be there for everyone who needs it without putting anyone at risk.

At this time, I didn’t feel like people were there yet. The wheels were just starting to turn.

I had my last in-person doctor’s appointment on March 6th, followed by the last in-person shabbat service the next day. I remember looking around the sanctuary and wondering when we would return and who would be there. It was like a scene from a Hitchcock film where the threat is unclear lurking just under the surface.

A few days later, there was a dress rehearsal for a play at my child’s school. Surely this virus had to be among us at that point. It had been discovered not far from where we lived just a few days earlier. As I separated myself in the auditorium and watched the dress rehearsal, I knew Les Misérables would never premiere. Opening night of the play was canceled. School was canceled. Our soup kitchen shuttered because it was too dangerous.

I found myself in full swing and ready to help. We made lists and reached out to others. Some were afraid to go grocery shopping, others wanted Lysol. Some needed help, and others had it under control. We had a great capacity of volunteers. More than those in need, which was a good position to be in. How could we leverage it? There was no model to follow. I made calls for another person to take over for me if I became ill.

Having pemphigus, it was too much of a risk for me to be around other people. But I had skills. I listened to what different organizations needed and how people wanted to help. I was the matchmaker. When a volunteer voiced a concern or needed something to change, I was ready. We brainstormed how to attack the problem and then made the pitch: we had many more people to help if only we could fix this problem. Suddenly, we had leverage and developed partnerships. This continued day after day.

Having a rare disease prepared me for the COVID-19 pandemic. It kept me in tune with the news and gave me focus. As the year went on, I felt the haze lift, and I found that I had new anchors, new benchmarks. My volunteer day started when my work day ended. Some days, the two were woven together and longer than ever. The joy of empowerment was a high that bolstered me. I never left home, but I helped keep volunteers safe and guided them on their path. Maybe I hoped that if my own boat sank, someone would bail my hull. Maybe I wasn’t being altruistic, but realistic. Pemphigus has made me this way—for better or worse.

This past year, we have fed thousands; collected personal protective equipment, diapers, food, toys; and created a system for getting seniors signed up for vaccines. By being flexible and listening to what people need, we continue to lend support to our community—one person and one need at a time.

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Make no mistake, my position is one of great privilege. I have job security, as well as the ability to work from home and flex my time. I don’t live paycheck to paycheck. All of that is immeasurable. But I also think that none of what happened during the pandemic would have been possible without my being diagnosed with pemphigus. It has made me scrappy and creative. It has taught me not to take no for an answer and to find a solution. I can’t say I’m happy that I have pemphigus, but it has made me stronger, more self-assured, and sharper than I was before, even if on the surface people see me as overly cautious. And for all of these things, I am forever grateful.

Rebecca Oling, MLS, is a librarian with a background in Education at SUNY Purchase College. She is the author of several articles on instruction in libraries and more recently has begun to publish on instructional issues in the rare disease world.
The past year was challenging for most people. It was the first time in a century that the world dealt with a deadly pandemic. Hand sanitizer was used more often, but as a pemphigus vulgaris (PV) patient, I’ve carried hand sanitizer with me for the past 15 years. I’ve always been hyper-aware of the need to avoid anyone who is sick, but COVID-19 upended my usual plans. Now I was faced with a deadly virus that people were transmitting, many without even knowing that they were infected. In March of 2020, my life and my family’s life changed. The only way back to normalcy was through the development of a safe and effective vaccine.

As I reflect on the past year, there are multiple factors that led me to decide to get the COVID-19 vaccine. Early last year, I was living with PV, dealing with a mild flare, and receiving rituximab infusions. In the early part of 2020, I saw reports that described infections of a new respiratory virus in China. I am a pilot, and I follow reports like this as something that could potentially interfere with travel. The virus quickly spread, and by March many parts of the US were facing stay at home orders. I was due for another round of rituximab infusions in April, and the risk of traveling for work was too high. I made the difficult decision to take a leave of absence from my employer.

I didn’t know it at the time, but when I had dinner with my parents in March of 2020, it was the last time I would see them for a year. Like myself, my father and mother—78 and 81 years old respectively—are considered high risk. Throughout the pandemic, we followed public health guidelines and didn’t socialize with anyone outside of our households, including family members. I stayed busy at home and took every precaution if I had to leave.

Not long after the initial stay-at-home orders, I felt hopeful. Vaccine development had begun, and all the resources the world could muster were put behind it. Historically, the development of a vaccine took ten years to reach the general population, but it looked like we would have a vaccine in less than a year. The rapid development was due to a number of factors, such as past coronavirus vaccine research, scientific advances in vaccine technology, and massive funding and resources.

By the summer of 2020, a number of vaccines entered clinical trial phases. At this point, I began to watch vaccine development updates closely. As the trials progressed into phase 3, I thought more about what I would do as a PV patient. Would I be able to get the vaccine? Should I get the vaccine? Would it be safe? How would it affect my PV? Would I need to delay treatment?

Thanks to the IPPF Patient Education Series webinars and consultations with my physicians, I was able to come up with answers to these questions. I decided that I would get the COVID-19 vaccine when it was available to me.

In February 2021, I was finally eligible for the COVID-19 vaccine. In the past, it was routine to schedule vaccines like the annual flu shot. But this was different. When I finally had my appointment, it felt like I had won the lottery. My parents had already been vaccinated at this point, and my wife, a frontline healthcare worker, had also been fully vaccinated. Now it was my turn! The morning of my appointment, I was very excited. I felt like it was the light at the end of the tunnel. I received the Moderna COVID-19 vaccine, and I am now fully vaccinated. Though life hasn’t returned to normal, things are slowly moving in that
Based on the current information that is available, the IPPF Medical Advisory Council recommends that all people that have been diagnosed with pemphigus or pemphigoid and are considered immunocompromised receive a Covid-19 vaccination in conjunction with discussing their vaccine and treatment strategies with their physician.

David Baron is a pilot for a major airline in the United States. He lives in the Chicago area with his family.

The IPPF COVID-19 information page is updated frequently and is a valuable resource. Access it here: https://www.pemphigus.org/covid19
Pauline’s Story

Kevin Mead

Every patient story is unique. Though there are often common threads of lives changed, challenges faced, triumphs, and defeats, the uniqueness of each story is ever apparent. This story is one with the heroics of a comic book, the twists of a John Le Carré plot, and an outcome that is changing lives. Meet Pauline Menczer—like so many of you, a patient. But that is not even half of the story here.

Going back 20 years, surfing was a sport with a growing brand. Television technology allowed for better filming, and the lifestyle had an appeal to even those terrified by the smallest of waves. Australians were leaders in the sport, and in 1993, Pauline won the Women’s World Title. First twist, Pauline won as a rheumatoid arthritis (RA) patient. Second twist, Pauline claimed success as a gay woman in a sport not then known for its acceptance. Third twist, equality in sport, and especially in surfing, was just a dream at that time. Not only was Pauline denied the cash prize that the male champion received, but the trophy that she was awarded was actually broken! In fact, it wasn’t until 2019 that the World Surf League (WSL) sanctioning body offered equal prize money for male and female categories at their events.

In 2021, after about three years of development and production, Pauline had her story told as one of the surfers in the documentary film, Girls Can’t Surf. The film was released in Australia in March 2021 (where theaters are open). It is not yet distributed in the US, but you can watch the trailer at the link in the sidebar of this article. Pauline says that the reaction to the film

"I’d rather inspire people than make money."
has been both incredible and humbling. “There are gay kids using the film as a way to talk about them needing support in school,” she says, and it is “inspiring girls from all backgrounds.”

Believe it or not, Pauline is the only World Champion to have claimed the famed Bondi Beach in Sydney as their “home beach,” and there is now a movement to get a statue of her erected there. Pauline herself says, “If you can’t see it, you can’t be it,” and hopes that if the statue goes up it can serve as an inspiration for others to overcome challenges and strive to excel and succeed.

Pauline grew up facing many challenges. She was raised by a single mother of four after her father was murdered. In March, she told the Melbourne Age, “when other people threw stuff out, we would grab it and sell it in a garage sale.” On the day that she won her World Championship title, she could hardly walk to the water’s edge due to her RA, but as she says, “I seem to do really well under pressure.”

Pressure? Well, the next twist is pemphigus vulgaris (PV). “It took about eight months before I was diagnosed. I was diagnosed about two years ago. It’s been a rough two years,” she says. “But I finally found a good specialist in Sydney who put me on a high dose of steroids to start with, then I went on a clinical trial, which was not very successful, and I’m finally using rituximab that has finally given me some relief. I’m still taking steroids, but I’m hoping to completely stop using them in the next month or two.” Within the Australian health system, rituximab is covered for things like RA after a period of step therapy, but getting coverage for the drug to treat PV is more difficult. Pauline said that the pain of PV far exceeds that which she experienced with RA, and her new job means that she is prohibited from taking pain medications.

In the next twist, two fans in the US and Australia saw Girls Can’t Surf and started a GoFundMe campaign so that Pauline could get the prize money that she would have received in 1993 had gender parity been in place. That sum was $25,000, and the goal was reached. The excess funds were all paid forward by Pauline. She is directly supporting a pemphigus patient in the Philippines, and she has already sent $12,500 for life-altering medication and support. In addition, she has also donated a portion of the funds to a charity that supports surfing for the disabled and an Australian autoimmune foundation.

So, what’s next? First, Pauline is in the water she loves. “I have to wear a wetsuit and earplugs and everything to protect myself—but I am out there!” she says. The film has led to interviews and podcasts across Australia and around the world. She runs a Facebook page for Australian patients and wants to work on improving the impact of Rare Disease Day in Australia. She closes by saying, “I’d rather inspire people than make money.”

Kevin Mead joined the IPPF as an Executive Director in October 2020. He has over 25 years of experience in association management, fundraising, and project management.
Patients with rare, autoimmune skin blistering diseases such as pemphigus and pemphigoid often have painful blisters that may be exacerbated by stress. As a patient myself, my personal slogan is: Worry = Stress = Blisters.

Under normal circumstances, we have enough everyday stress to deal with. Since the early part of 2020, all of us have dealt with the added anxiety related to the COVID-19 pandemic. We may worry about whether or not we are more vulnerable to COVID-19 because of our immune system. Many patients have had concerns regarding the COVID-19 vaccines as well.

To address these concerns, there are resources available on the IPPF website, including information from our Medical Advisory Council and recordings of past webinars.

For many years, I have given a workshop on de-stressing at the annual IPPF Patient Education Conference. Due to the pandemic, the 2020 conference was held online, and I was unable to give my presentation. The following are some tips I typically discuss in my workshop that you may find beneficial in combating stress. A few of these methods will take practice. Take your time, take it easy, and know that it will get easier as you do them more often.

Meditation can give you a sense of calm and peace that benefits both your emotional well-being and your overall physical health. These benefits don’t end when your meditation session ends. Meditation can help you remain calm throughout your day.

As a patient myself, my personal slogan is: Worry = Stress = Blisters.

Keep in mind that when you close your eyes and look straight ahead (the area between your eyebrows), it keeps your mind active, and it will be easier to reach a meditative state. If you look down, you may fall asleep!
Remember—it does take practice, so don’t feel bad if you get sleepy in the beginning.

When your eyes are closed, imagine that you are in a wonderful place that you enjoy. It could be the beach, the mountains, a favorite hiking trail, a lake, or just lying on the grass looking up at the sky. The more comfortable you feel, the more relaxed you will be.

**Deep Breathing.** I believe that deep breathing is always helpful. It’s like a tonic for your heart and gives you a sense of well-being. To practice, inhale to the count of 20 (or 5 or 10 to start), hold for 20 seconds (or 5 or 10) and exhale for 20 seconds (or 5 or 10). Try doing this several times with your eyes closed. Each time you inhale and exhale, you should become more relaxed. You can also add words like inhaling to “joy” and exhaling to “peace” or whatever works for you.

**Listening to calming music** is a helpful technique when dealing with stress. Meditation, yoga, or classical music can help you relax.

**Watching comedies or documentaries** may also help reduce your stress levels. I find that having a cup of tea and watching a travelogue is always great for my mind and helps me imagine that I’m somewhere else.

**Yoga** is good for both the body and mind. There are a variety of free resources on practicing yoga online to help strengthen your body.

If you feel anxious and find yourself worrying about something in particular, I recommend reaching out to talk to someone, such as a family member, minister, bishop, rabbi, therapist, or friend. If your feelings are bottled inside of you, this may lead to more stress and affect the physical symptoms of your disease. I hope you find these techniques helpful. Feeling calm and remaining in control of how your mind and body work together will lead you to a better place.

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**Mei Ling Moore** was diagnosed with PV in 2002. She has been a peer health coach with the IPPF since 2012. She also organizes the Southern California support group with Marc Yale. Mei Ling lives in Los Angeles.